

Inpatient Admissions and Emergency Department Visits for Patients with Facioscapulohumeral Muscular Dystrophy (FSHD): A Real-World Retrospective Data Analysis of Pre- and Post-Diagnosis Events



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Objectives

Describe the changes in inpatient admissions and emergency department visits in patients with FSHD compared with matched controls (MC) 2 years post-diagnosis versus 2 years pre-diagnosis

Background

- FSHD is a rare, slowly progressive, genetic skeletal muscle disease. Muscle weakness usually presents in the face and upper extremities, eventually extending to the trunk and lower body^{1,2}
- Patients experience significant physical limitations, pain, fatigue, and an overall negative impact on wellbeing.^{3,4} Real-world data characterizing the patients' pre-diagnosis journey are limited
- Currently there is no cure or targeted treatment for FSHD⁵

Methods

- Retrospective database analysis to compare outcomes for patients with FSHD versus MCs
 - Database: IQVIA US PharMetrics® Plus Timeframe: January 2016 through March 2021
- The FSHD cohort is defined as having ≥2 FSHD claims ≥30 days apart
 - Claims identified by International Classification of Disease, Tenth Revision (ICD-10) code G71.02
 - The first diagnosis date was used for the index date
- FSHD patients were matched to a 5% random sample of eligible non-FSHD controls
 - Matching was done using R's Matchit procedure, with nearest neighbor matching (exact matching on month of index date)
- Cohorts were matched (5-MC:1-FSHD) on index month and baseline age, region, gender, plan, and payer types
- All subjects (patients and MCs) had a minimum of 48 months of continuous data:
 - 24 months prior to their index date
 - 24 months following ("post") their index date (includes the index date)
 - The index date was the beginning of the post-index evaluation period
- Costs were inflation-adjusted using the US Bureau of Labor Statistics' medical cost Consumer Price Index (CPI) for December 2020
- For the inpatient admissions and emergency department visits, the following were compared: pre-, post- and the changes (post-pre):
 - The percent of each cohort with claims (at these locations)
 - The costs, number of services billed, and days of service
 - The most common reasons for inpatient admissions and emergency department visits
- Comparisons were made using t-tests for continuous variables and chi-square tests for discrete variables
- Post-pre changes were compared within cohorts (using McNemar tests) and between cohorts (using t-tests)

Results

- We identified 79 FSHD patients and 395 MCs
 - There were no significant differences between cohorts for age, gender, US region, patient plan type, and patient insurance type (Table 1)
- The cohorts had significant ($p < 0.05$, except where noted) differences for the Charlson Comorbidity Index (Table 2)
- Within the FSHD cohort, the following locations of care had significant ($p < 0.05$) increases in outcomes:
 - The difference in percent of patients seeing the emergency department was 16.5% (primarily due to increases in respiratory condition visits)
 - Total annual medical costs per person increased \$5,646
- FSHD patients had higher inpatient admissions and emergency department visits:
 - FSHD patients had higher utilization of care in the inpatient setting (Table 3) and emergency department (Table 4) as measured by the percent with claims and mean costs, services, and days of service
 - The admission diagnoses by Agency for Healthcare Research and Quality (AHRQ) category pre- and post-index for FSHD patients and MCs are shown for inpatient admissions (Figure 1) and emergency department visits (Figure 2)

Table 1: Age, Region, Insurance, and Payer Types Were Similar Between FSHD Patients and MCs

Descriptive Characteristics	FSHD Patients (N=79)
Gender, (% female)	43.0%
Age, years mean (SD)	47.9 (17.9)
Age, years	
<18	6.6%
≥18 to <35	17.6%
≥35 to <45	11.0%
≥45 to <55	22.4%
≥55 to <65	30.3%
≥65	12.1%
US region	
South	33.1%
Midwest	30.0%
Northeast	20.3%
West	16.6%
Insurance type	
Preferred provider organization	61.7%
Health maintenance organization	29.3%
Point-of-service plan	1.0%
Consumer-directed healthcare	5.9%
Indemnity/traditional plan	2.1%
Payer Type	
Commercial	61.7%
Self-insured	29.3%
Medicaid	1.0%
Medicare Advantage	5.9%
Medicare Supplemental	2.1%

*Difference significant at $p < 0.05$

Table 2: Before and After Diagnosis, Charlson Comorbidity Index Scores Were Higher for FSHD Patients Versus MCs

Descriptive Characteristics	FSHD Patients (N=79)	MCs (N=395)
Pre-index (before diagnosis)		
Mean (SD) score	1.13 (1.65)	0.74 (1.67)
Percent with values >1*	27.8%	14.2%
Post-index (after diagnosis)		
Mean (SD) score*	1.37 (1.65)	0.82 (1.73)
Percent with values >1*	29.1%	17.5%

*Difference significant at $p < 0.05$

Abbreviations:

AHRQ, US Agency for Healthcare Research and Quality; COPD, chronic obstructive pulmonary disease; CPI, Consumer Price Index; FSHD, facioscapulohumeral muscular dystrophy; ICD-10, International Classification of Diseases, Tenth Revision; MC, matched control; PMPY, per member per year; SD, standard deviation.

References:

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Conclusions

- The journey to a FSHD diagnosis identified higher use, cost, services, and days in inpatient care and emergency department visits prior to the FSHD diagnosis
 - This likely reflects the need to investigate and manage previously unsuspected manifestations of FSHD following formal diagnosis
- Future research should confirm if these findings hold true in longer-term follow-up
- These data highlight the many unmet needs for FSHD patients, including higher costs, more days of care, more prevalent and costly comorbidity management, and the need for novel targeted treatments
- Based on the high unmet need, Avidity Biosciences is planning clinical trials with a first-in-class antibody oligonucleotide conjugate targeting DUX4, the underlying cause of FSHD, in 2022

Table 3: Utilization of Care for Inpatient Admissions

Outcomes, PMPY	Pre-Index		Post-Index	
	FSHD Patients	MCs	FSHD Patients	MCs
% with claims	22.78%*	11.14%	30.38%*	10.13%
Costs, mean (SD)	\$3,048 (\$10,059)	\$894 (\$3,849)	\$3,856 (\$11,596)	\$1,690 (\$8,351)
Services, mean (SD)	3.18 (7.39)	1.48 (7.23)	5.99 (23.50)	2.24 (11.23)
Days of service, mean (SD)	0.56 (1.41)	0.25 (1.24)	1.85 (8.92)	0.39 (2.14)

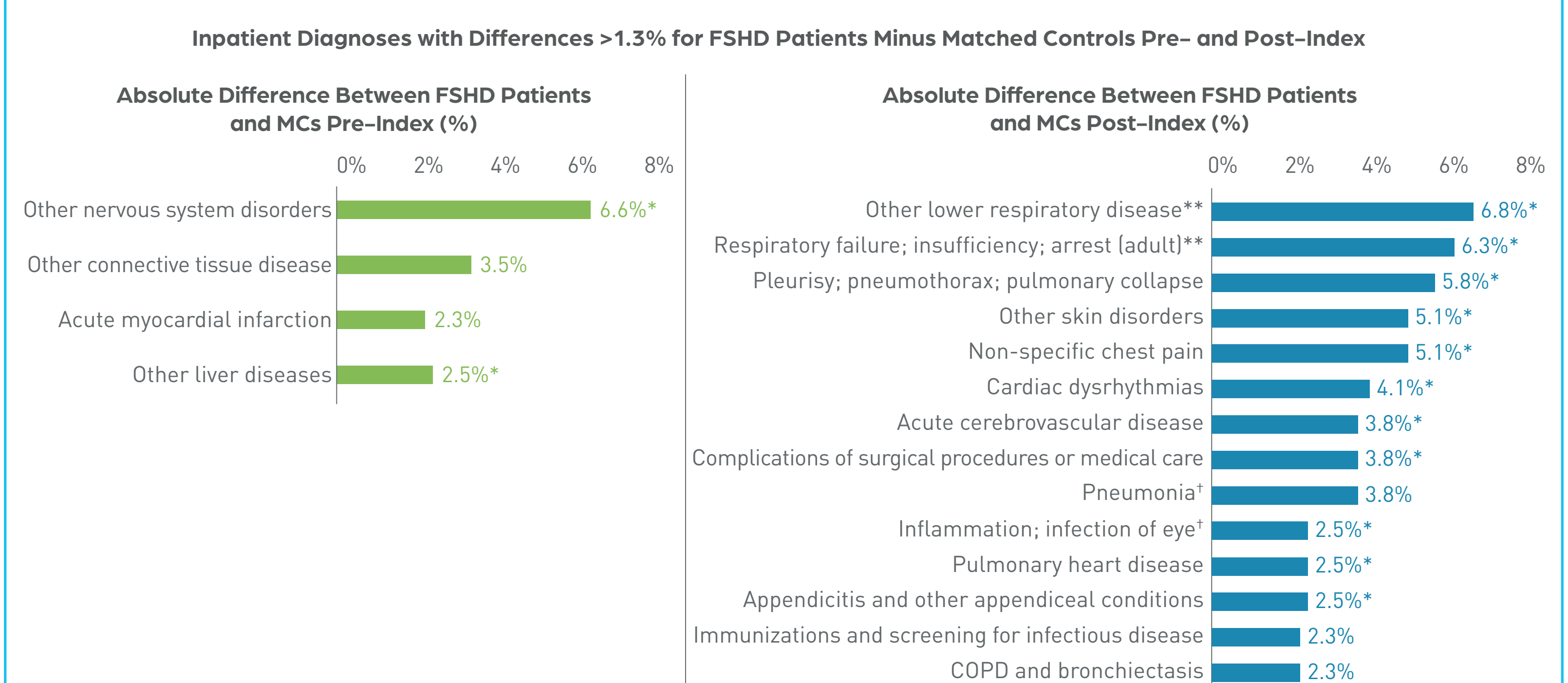
Significant differences: between cohorts * $p < 0.01$

Table 4: Utilization of Care for Emergency Department Visits

Outcomes, PMPY	Pre-Index		Post-Index	
	FSHD Patients	MCs	FSHD Patients	MCs
% with claims	17.72%	15.70%	34.18%*†	18.48%
Costs, mean (SD)	\$230 (\$1,033)	\$141 (\$780)	\$249 (\$704)	\$205 (\$1,390)
Services, mean (SD)	1.21 (4.25)	0.83 (3.73)	1.50 (3.14)	0.88 (3.67)
Days of service, mean (SD)	0.23 (0.66)	0.17 (0.50)	0.37 (0.71)*	0.16 (0.48)

Significant differences: between cohorts * $p < 0.01$
within cohort change (post-pre) † $p < 0.05$

Figure 1: Difference in Inpatient Admission Diagnoses by AHRQ Category Pre- and Post-Index for FSHD Patients Minus MCs (All $p < 0.05$)



* $p < 0.01$

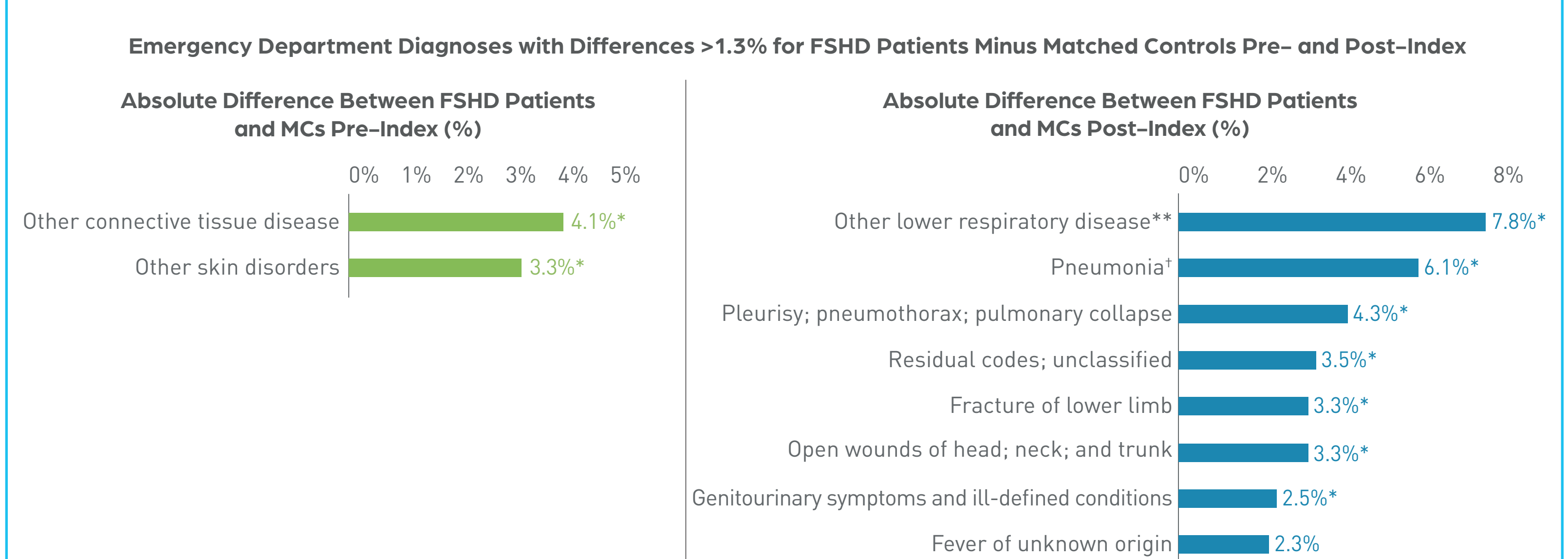
**Inpatient "respiratory failure; insufficiency; arrest (adult)" was the only category with a statistically significant ($p < 0.05$) difference:

- Between FSHD patients and MCs post- minus pre-index, with a difference of 6.6%

- The (post-pre) change within the FSHD patients

†Except that caused by tuberculosis or sexually transmitted disease

Figure 2: Difference in Emergency Department Diagnoses by AHRQ Category Pre- and Post-Index for FSHD Patients Minus MCs (All $p < 0.05$)



* $p < 0.01$

**"Other lower respiratory disease" and "pneumonia" were the only categories with statistically significant differences between FSHD patients and MCs post- minus pre-index, with differences of 7.6% and 6.6% respectively ($p < 0.05$)

†Except that caused by tuberculosis or sexually transmitted disease